

Patient-Focused Drug Development: Understanding the Patient Experience and Meaningful Treatment Outcomes in Becker Muscular Dystrophy via Qualitative Interviews

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Conclusions

- Qualitative interviews facilitate a deeper understanding of patient experiences and are used as a method to contextualize the meaningfulness of treatment outcomes.
- ARCH and CANYON participants described the various symptoms and the impacts of Becker muscular dystrophy (BMD)
- Improvements in symptoms/impacts as well as disease stabilization or slowing of progression are acknowledged as meaningful treatment benefits to individuals living with BMD.
- These interviews provide support for the relevance of functions assessed in the North Star Ambulatory Assessment (NSAA) to BMD patients.



Background

Becker muscular dystrophy (BMD) is a serious, rare neuromuscular disorder with no approved therapies. Sevesamten is an investigational, novel, oral, fast skeletal myosin inhibitor designed to protect muscle against contraction-induced damage while preserving function.¹

Two clinical trials, ARCH (NCT05160415) and CANYON (NCT05291091), were conducted as part of the BMD clinical development program of sevesamten.^{2,3}

In these trials, qualitative interviews were conducted with a subset of clinical trial participants to gain a deeper understanding of the lived experience of BMD patients and to assess if treatment effects address aspects of the disease that are most relevant and meaningful to patients.

Methods

Study Design

- ARCH (NCT05160415): open-label study of sevesamten, assessing safety, pharmacokinetics, and biomarkers in adults with BMD.
- CANYON (NCT05291091): Phase 2, double-blind, placebo-controlled study of sevesamten, assessing safety and efficacy in adults and adolescents with BMD.
- Exit interview results focused on understanding BMD symptoms and the degree in which outcome measures assess these symptoms.
- For the purposes of this poster, results from the 2 clinical trials were aggregated, where possible.

Table 1. Select demographics and trial details

	Interviews (n)	Age at time of interview, years (M, range)	Key Clinical Trial Inclusion Criteria
CANYON	25	27.8 (15-52)	<ul style="list-style-type: none"> Ambulatory adult and adolescent males with BMD Age 12-50 years old NSAA score between 5-32
ARCH	8	35.1 (24-49)	<ul style="list-style-type: none"> Ambulatory adult males with BMD Age 18-55
Total	33	29.5 (15-52)	-

M = mean; NSAA = North Star Ambulatory Assessment.

Interviews

- 1-hour interviews
- Conducted following the completion of treatment (ARCH) or within 5 days ahead of completing treatment (CANYON)
- Participants and interviewers were blinded to treatment for CANYON interviews.

Semi-structured discussion guides were designed to assess:

- BMD signs, symptoms, and impacts prior to the study
- Most bothersome symptoms and impacts
- Meaningful treatment benefits
- Relevance of items assessed by the NSAA (ARCH only)

References

- Russell AJ, et al. J Clin Invest. 2023;133(10):e153837.
- Clinicaltrials.gov. 2025. <https://clinicaltrials.gov/study/NCT05160415>.
- Clinicaltrials.gov. 2025. <https://clinicaltrials.gov/study/NCT05291091>.

Sevesamten is an investigational agent that is not approved for use by any regulatory authority in any territory.

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Results

Pretrial BMD Signs, Symptoms, and Impacts

The most commonly reported signs and symptoms experienced prior to starting the studies included:



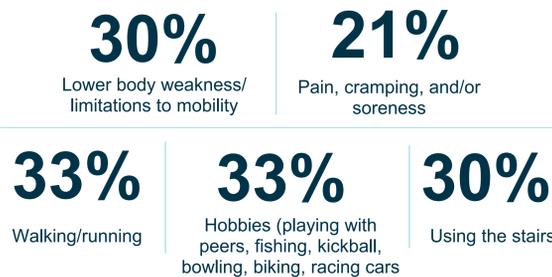
Further, most participants reported that their BMD symptoms impacted their



“ It’s just the cramping... It’s kind of burning, [is how] I would describe it. But I don’t know, it’s just like you’re weaker, you’re more tired. I feel like a normal person would take a while to get that; I got that in an hour of exercising. ”
(ARCH participant)

“ I will say that if something’s heavy, I can’t really lift it mainly because the lower part of my body is too weak, and what will happen is I can’t either...I can’t keep steady as I’m walking with it, or I can’t get it up in the first place because I can’t bend down and get back up without pushing off something with my hands. ”
(CANYON participant)

Most Bothersome Reported BMD Symptoms and the Most Reported Activity Impacts



“ I guess another one is I used to be really good at skiing and now I can’t really do that anymore. But that’s not as much, that’s like a day-to-day activity. But stairs is probably the biggest and most common thing, so that would be why it’s bigger for me. ”
(ARCH participant)

“ I think for me, definitely it [Becker] affecting my core and then my quads. I think in terms of the functional thing, I think walking is the most important or the thing that I notice the most because you need to walk to your car or whatever. I don’t use a mobility device. I’m just walking independently. So I think that’s something that I feel the most with this. ”
(CANYON participant)

Relevance of the North Star Ambulatory Assessment

ARCH participants (n=8) were asked to describe the activities impacted by their limitations in carrying out the physical functions assessed by the NSAA. In total, all but 1 of the physical functions (lifting head from lying) assessed by the NSAA were relevant to at least half of participants:



Functional Stability and/or Slowing of Disease Progression as Meaningful Outcomes

Participants in both studies were asked to describe the type(s) of treatment outcome(s) that would be meaningful to them. Although participants indicated that improvements in functioning or reversing the effects of BMD would be ideal, among those asked (n=26), all (100%) noted that functional stability or slowing of disease progression would be an important and meaningful treatment outcome.

“ So seeing them [symptoms] slow down or stop in decline is just about the only thing that I want to see, I guess. So that’s the main goal, I guess. ”
(ARCH participant)

“ I suppose I really want to get better obviously. And I know that everybody probably wants that. I don’t know how possible that is. But yeah, definitely not getting worse is important. ”
(CANYON participant)

“ I think anything that can keep me at the level that I am right now, that can slow down progression. It would always be awesome if they came up with some kind of drug that improves my conditions. But I think, mainly, it’s a thing of keeping me balanced, and keeping me kind of at the level that I am right now. ”
(CANYON participant)